

WINTER 2012-2013

the Advocate

Your connection to the Friedreich's Ataxia Research Alliance



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FARA

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Working Together, We Will Succeed

By Ron Bartek

Dear friends,

I am so proud of our FA family! As you can see in this issue, we have come a very long way together over the last five years. The FARA treatment pipeline now contains far more candidates and a number of them are much closer to the finish line. And, as you know well, this has not been by accident. We have not simply ridden a fortunate wave of progress. Rather, working hard together, we have helped create that wave. Our tremendous progress is due primarily to the partnerships and family relationships we have built and nurtured with each other and with the FA scientists, the National Institutes of Health (NIH), the Food and Drug Administration (FDA), drug companies, and other patient advocacy organizations around the world which have placed FARA and the FA family in a real leadership role. Because we are increasing the number of potential FA therapies prepared to enter clinical trials, it has become more and more important to work effectively with the FDA.

FARA is actively engaged on two important fronts to help the FDA reach sound, prompt judgments regarding the safety and efficacy of potential FA therapies. First, we are taking advantage of every opportunity to deepen the FDA's understanding of FA — the severity of the disease, its impact on quality of life, and the lack of treatment — so the agency hears the voice of FA patients and is better prepared to make appropriate benefit-risk evaluations at every step in the regulatory process. Second, we have joined with a broad coalition of advocates from every FDA-regulated sector in a concerted effort to convince the U.S. Congress to provide the agency with the resources it needs to do its job quickly and well.

On the educational front, FARA assembled and led a coalition of advocacy organizations that submitted to the FDA a recommendation to include FA and a cluster of other degenerative neuromuscular disorders as one of the 20 groups with which the

agency is to meet over the next five years. You can see that recommendation on the FARA website at http://curefa.org/_pdf/FDA-publicComments11-01-12.pdf. FARA is also joining a growing number of pharmaceutical partners in their initial FDA meetings to initiate clinical trials. In these meetings, we are explaining FA and the importance of each trial from the FA patient's perspective. At the other end of the regulatory pathway, the FDA recently appointed the FA community's first patient representative to provide input to the committees that consider submissions for market approvals of therapies following completion of their clinical trials.

On the resources front, FARA continues to be an active member of the Alliance for a Stronger FDA as we and our Alliance partners remind each member of the U.S. Congress that the chronically under-funded FDA must be given the resources it needs to advance the safe, effective therapies our patients need so badly. I always tell these congressional offices that a stronger FDA will save our patients' lives while a weaker FDA will threaten them. FARA remains the spotlighted patient advocacy member on the website of the FDA Alliance at <http://strengthenfda.org/members/member-spotlight/>.

Please join in helping the FDA, and our other major government agency ally — the NIH — and you will help all of us. You have probably received FARA's appeal to communicate with your congressional representatives the importance of protecting the FDA and NIH from budget cuts ("We need cures not cuts!"). You can find on the FARA website at www.curefa.org very helpful materials and templates for your communications with your congressional representatives.

You can see why I am so proud of the FA community for all we have done together to make such progress over the last five years. At the core of the progress is the individual FA family — the reason we all work so hard. We have much yet to do together. However, I know we will get it done and I know it is your hard work, commitment, and support that will continue to empower and enable the FARA family to lead the way in accomplishing the goal we all share — to treat and cure FA.

Thank you and warm regards,
Ron



FARA's Path Forward 2015

By Jennifer Farmer

It is hard to believe that I have been in my role as Executive Director of FARA for five years. Some days it seems like yesterday that I was a new genetic counselor, working in neurogenetics, who shared a diagnosis of FA with a family and drew blood samples for our first research studies on iron abnormalities in individuals with FA. I drew pictures of chromosomes and genes for Ron and Raychel Bartek as we discussed the significance of identifying the gene that causes FA while Ron and Raychel told me about a new organization they were establishing to advance research and find treatments. This meeting occurred in 1998!

Time is not on our side. Everyone at FARA understands this deeply and personally. There must be urgency in our pace and laser focus on our mission.

This year FARA staff and directors reflected on our progress against our first strategic plan, which we implemented in 2008. We evaluated our current environment to create a new plan that will carry us forward to 2015. The purpose of a strategic plan is to provide clear direction and focus for an organization, setting goals and priorities that ensure that we achieve our mission – treatments and a cure for FA.

We first started using the visual representation of the treatment pipeline in 2007. This pipeline conveys important concepts about our initial strategy:

Diversify – Rarely is any disease treated with only one drug or one approach. So, based on understanding of the disease mechanisms, we need different approaches to discover different types of therapies. Along the bottom of the chart are mechanisms of action or treatment approaches.

Multiple shots on goal – Since drug development is very risky and has a high failure rate for a variety of reasons, including

safety, tolerability, efficacy, etc., we need multiple candidates for each mechanism or approach.

It is quite striking to look at 2007 compared to 2012. It is immediately obvious that there are more therapeutic approaches and new drugs in the pipeline. This is a direct result of our understanding the underlying cause of the disease and pursuing more targeted research for drug discovery. In fact, as I prepare this article and update the pipeline, two FARA-funded scientists working on drug discovery projects (depicted in the bars on the far right) reported on their work at the Ataxia UK Scientific meeting. Both scientists have identified new compounds (with different mechanisms of action) that increase frataxin in cellular assays and models. These new candidates are in the process of further validation and will likely be reflected in the next year's pipeline as new bars. It is essential that we have new candidates populating the pipeline.

A comparison of the two charts shows some important breakthroughs and milestones:

Idebenone – Phase 3 trials completed and approved in Canada

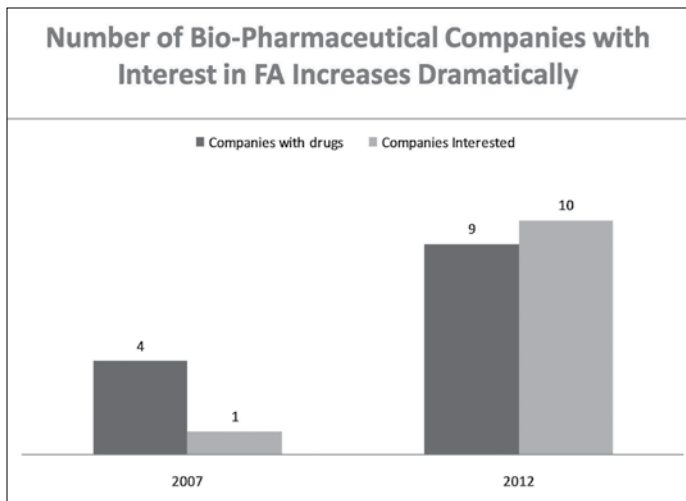
EPI-A0001 – Completed Phase 2a trial in which improved neurologic function was observed. A “sister” compound, EPI-743, which has been studied in several different mitochondrial diseases, will soon launch a Phase 2 trial in FA.

HDAC inhibitors – A human clinical trial for the first HDAC inhibitor, RG2833, began in Italy in March 2012 and is ongoing. Other HDAC inhibitors such as RG3250 are advancing through pre-clinical development, and a clinical trial of Nicotinamide has begun in London.

Gene therapy – Proof-of-concept of AAV-based gene therapy shows potential for a significant benefit since it completely reverses phenotype in cardiac knock-out mice.

On our website is a detailed overview of the FA treatment pipeline with updated information on all of the candidates.

Another important development is growing interest from and partnership with bio-pharmaceutical companies. Many of the new bars on the pipeline represent new drug candidates under development by companies who have identified Friedreich's ataxia as a disease area of interest. This year a growing number of



bio-pharmas contacted FARA to express their interest in FA. The chart gives you a sense of the magnitude of this interest. Some companies have compounds or technologies that they think will be of potential benefit for FA. We are working closely with companies like ViroPharma, Retrotope, and Statagics as they work through pre-clinical development and plan for clinical studies. Other companies have identified FA as an area of interest and seek partnerships with discovery scientists who have candidates for advancement and will benefit from the infrastructure and experience of a drug development partner.

In asking such companies “What attracted you to FA?”, the responses include:

- *There is a lot of good basic research that has informed the cause of the disease. A lot is known about the frataxin gene, disease mutation, and protein. There are animal and cellular models of the disease that can be used to screen and test compounds.*
- *The clinical infrastructure exists to inform, design and conduct clinical trials. FARA's Patient Registry is the only international registry of individuals with FA with more than 1,800 individuals enrolled. One of the primary concerns facing a pharmaceutical company considering clinical trials of a drug in a rare disease is whether we will be able to recruit enough patients for clinical trials. By having an active patient community that is interested in clinical trials and the infrastructure (the patient registry) in place to quickly communicate with patients, we can significantly reduce the time and effort required for clinical trials. The Collaborative Clinical Research Network in FA works with pharmaceutical partners to help them understand the clinical aspects*

of the disease, the natural history, and to help design and plan trials based on clinical outcome measures and biomarkers that have been validated.

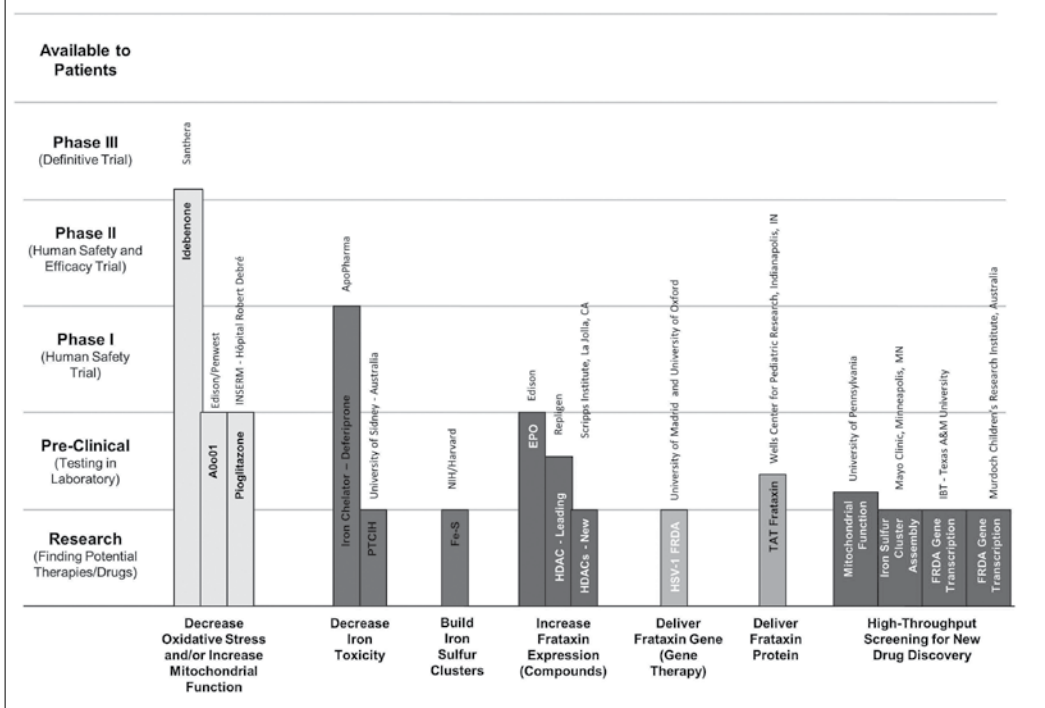
- *FARA directly funds some of the research through grants for pre-clinical and clinical development. The resources needed to get drugs through pre-clinical and clinical development are significant – estimates range from \$5-15 million, depending on the drug candidate. Providing some of the funds, especially at earlier stages of development, can help smaller companies get started and do the necessary studies.*
- *Friedreich's ataxia can be a model for developing treatments for other more common complex diseases. Some of the disease mechanisms and cellular pathways that are affected in FA are also affected in other neurodegenerative and mitochondrial conditions.*

FARA's Path Forward 2015 reflects our adjusted goals with a very aggressive strategy for the treatment pipeline. We want to have a mitochondrial function/energy metabolism therapy approved and available to patients. Since reduced levels of frataxin cause the disease, we have to bring more candidates to the pipeline that increase, stabilize, or substitute for frataxin. We will pursue advances in gene therapy and exploration of stem cells as a treatment to replace damaged tissue.

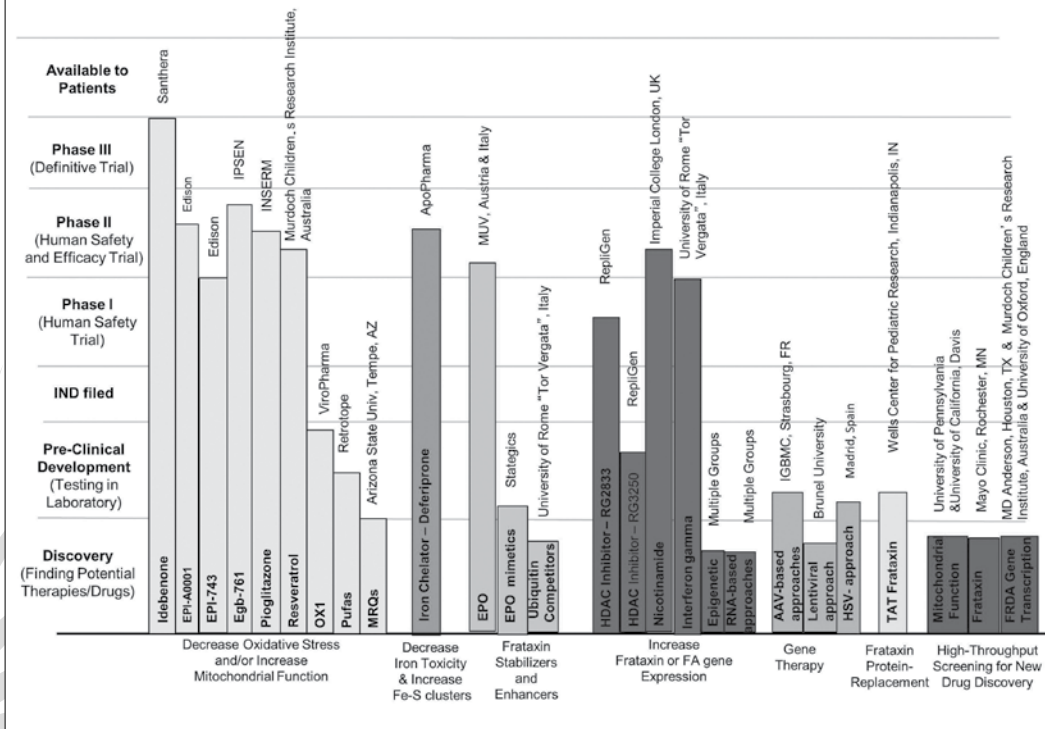
Over the past five years FARA has expanded its staff, directors, and advisors so that the necessary expertise, human resources, and capacity are in place to move with greater urgency, affect more progress in research, and overcome some of the hurdles that exist in the business of finding treatments and ultimately a cure for FA.

While it is taking longer than any of us would like, there have been significant accomplishments, advancements, and breakthroughs. We have expanded our network of partnerships with biopharmaceutical companies and increased our internal capacity. With this progress we face new challenges, which we are ready to take on. I am continually energized by all the people in FARA – the staff, directors, ambassadors, volunteers, scientists, partner organizations, and those who battle FA with strength, courage, and determination. ●

FA Treatment Pipeline - 2007



FA Treatment Pipeline - Oct 2012



A Review of FARA's Research Grant Program

By Bronya Keats, MD

Exciting research funded by FARA continues to move us closer to effective treatments for FA. Over the past 12 months, FARA reviewed 24 new grant applications and awarded funding to 12. FARA is particularly pleased that the Bob Allison Ataxia Research Center (BAARC) at the University of Minnesota agreed to co-fund the Phillip Bennett and Kyle Bryant Translational Research Award to Dr. Isabelle Iltis (see summary of this grant and other recently funded grants below). In addition to the 12 new grants, FARA approved continuation funding for 14 projects. Total research funding awarded so far during 2012 is approximately \$2.9 million, and up to four more grant applications are likely to be funded before the end of the year. We actively seek co-funding from our partners, such as BAARC in order to increase the number of available research dollars and minimize duplication of effort.

These funded projects address numerous important research questions that target FARA's research priorities, including advances in differentiation of induced pluripotent stem (iPS) cell lines from people with FA into sensory neurons and cardiomyocytes, development of new drugs that improve mitochondrial function in people with FA, improvements in functional measures and new biomarkers for clinical trials, advances in cell and gene therapies, and evaluation of candidates to advance to the treatment pipeline. In addition to research projects, FARA continues to support and expand the Collaborative Clinical Research Network (CCRN) and the standardized characterization of FA mouse models at the Jackson Laboratory.

Among the many important contributions to the literature resulting from FARA funding include a recent report from Dr. Arnulf Koeppen (see "Featured scientist") on "The relation of cytosolic iron excess to cardiomyopathy of Friedreich's ataxia." This discovery shows that iron-mediated damage to cardiomyocytes and myocardial scarring are more likely to be due to iron excess in the cytosol rather than selective iron overload only in cardiac

mitochondria as previously suggested. Dr. Koeppen performed his studies using a combination of X-ray fluorescence, slide histochemistry of iron, and immunohistochemistry of two iron-related proteins to examine human cardiac tissues from the left and right ventricular walls, ventricular septum, right atrium, and atrial septum. (Ramirez RL, Qian J, Santambrogio P, Levi S & Koeppen AH. Am. J. Cardiol. Published online 24 Sept 2012).

In another recent report of FARA-funded research, Drs. Gary Rance, Louise Corben and Martin Delatycki demonstrated that "Auditory pathway changes mirror overall disease progress in individuals with Friedreich ataxia" in a longitudinal study of two males over a three-year period. These results are consistent with previous cross-sectional studies and suggest that hearing measures may serve as indicators of generalized neurological change. Auditory tests have fewer limitations than examination-based scales (such as FARS) and may be useful clinical biomarkers. (Rance G, Corben L & Delatycki MB. J. Neurol. Published online 30 Sept 2012)

A comprehensive list of FARA-funded and other research publications on FA is available at: <http://www.curefa.org/scientific-news.html>

Recently Awarded Grants

Mass spectrometric analysis of metabolic features of Friedreich ataxia

Principal Investigator: Dr. David Lynch, Children's Hospital of Philadelphia, PA

Dr. Lynch is collaborating with Dr. Ian Blair to advance understanding of metabolism in FA. To determine if changes are found in the metabolomic profile of FA cells, they propose using a powerful and rigorous mass spectrometry technique developed by Dr. Blair to monitor metabolic changes revealed by intracellular levels of various short chain acyl-CoA thioesters. In preliminary studies, Drs. Lynch and Blair have found that decreased frataxin expression results in changes of intracellular levels of specific CoA thioesters, most notably a decrease in the intracellular acetyl-CoA to succinyl-CoA ratio. This same change was observed in platelets from FA subjects. They have also developed a method to monitor changes in metabolic flux (the rate of turnover of molecules

through a metabolic pathway). By expanding these methods to a broader set of intracellular metabolites, a more thorough characterization of the metabolic changes in frataxin deficiency can be achieved. Metabolic flux through central and alternative metabolic pathways will also be assessed. These studies will identify potential biomarkers of disease, and they may also guide novel pharmacological and nutritional interventions that complement other treatment paradigms.

Role of mismatch repair enzymes in Friedreich's ataxia repeat expansion pathogenesis

Principal Investigator: Dr. Joel Gottesfeld, The Scripps Research Institute, La Jolla, CA

Based on the hypothesis that the expanded GAA repeat (common mutation that causes FA) changes the acetylation state (tags that control gene expression) of FXN chromatin might be responsible for gene silencing, the Gottesfeld lab identified a specific class of HDAC inhibitors, which target this acetylation state, as potent activators of FXN transcription in cell culture. These molecules have been found to cross the blood-brain barrier and increase FXN mRNA and frataxin protein levels in the brain and heart in mouse models for FA. During previous studies they found that these molecules that specifically target HDAC3 do not restore FXN gene expression in FA cells (lymphocytes and iPSC-derived neurons), while molecules that are moderately HDAC3 selective are active. They also found that the mismatch repair enzymes MutS α and MutS β are direct targets of the HDAC inhibitors. Mismatch repair enzymes are special enzymes that can recognize and repair errors in the DNA. The purpose of this project is to explore the physiological significance of these findings and probe the role of the mismatch repair machinery in FA pathogenesis. These results will shed light on the mechanisms of gene silencing in FA and will guide therapeutic development.

Phillip Bennett & Kyle Bryant Translational Research Award Mapping the anatomical and functional connectivity of the central nervous system in Friedreich's ataxia using magnetic resonance imaging

Principal Investigator: Dr. Isabelle Iltis, University of Minnesota
Dr. Iltis and her co-investigator, Dr. Christophe Lenglet, are using magnetic resonance imaging (MRI) techniques to advance understanding about the connections between the different structures of the brain and spinal cord in Friedreich's ataxia. These

techniques offer a non-invasive approach to observing and assessing the integrity of the central nervous system's connectivity in-vivo (in individuals with FA). Spinal cord diffusion MRI has not previously been performed in people with FA. Because spinal cord atrophy with dorsal root ganglion degeneration is a hallmark of the disease, significant differences in the integrity of spinal pathways may be identified and represent potential specific biomarkers. The same techniques will allow the detection of altered pathways from/to the cerebellum, and more specifically the dentate nucleus, and structures such as the superior cerebellar peduncles and motor cortex.

Titles and summaries of most of the projects presently funded by FARA are available at:

www.curefa.org/RPMP/public/pggrantlist.aspx and complete listings of grants awarded by year can be accessed at: www.curefa.org/grants-awarded.html

New grant application guidelines

Beginning January 1, 2013, new changes to the grant program will be in effect. A letter of intent will be required for all grant proposals and submission of a full application will be by invitation only. Additionally, there will be deadlines for each grant type as follows:

Grant Type	LOI Deadlines	Application Deadlines	Maximum Budget (in US dollars)
General Research Grant	February 1 July 15	April 1 September 15	\$150,000 per year for 1 or 2 years
Keith Michael Andrus Cardiac Research Award	January 1	March 1	\$150,000 per year for 1 or 2 years
Phillip Bennett and Kyle Bryant Translational Research Award	May 15	July 15	\$250,000 per year for 1 or 2 years
Bronya J. Keats International Research Collaboration Award	May 15	July 15	\$200,000 per year for 1 or 2 years

Exceptions to these deadlines are projects that the investigator believes may be of high priority to FARA. An LOI for such a proposal may be submitted at any time during the year. However, the justification for such special consideration must be compelling.

The complete new guidelines will be available on the FARA website by January 1. ●



Dr. Arnulf Koeppen

By Felicia DeRosa

Due to his clinical and research experience in Friedreich's ataxia (FA), Dr. Arnulf Koeppen understands the need to collaborate with physicians in several medical specialties. Patients with FA suffer from disorders of dorsal root ganglia, spinal cord, nerves, brain, eyes, heart, and the insulin-producing cells of the pancreas. His goal is effective treatment and, ultimately, a cure of FA. A neurologist and neuropathologist at the VA Medical Center in Albany, NY, and Albany Medical College, Dr. Koeppen is also a FARA Scientific Advisor. He has spent several years studying iron metabolism in FA and he leads the Tissue and Organ Donation program.

Dr. Koeppen first developed an interest in hereditary ataxia in 1973, when he was a consultant for his orthopedic colleagues. A patient with hereditary ataxia had broken his ankle during a fall, and his surgeons wanted to learn about ataxia causing the accident. In 1980, Dr. Koeppen learned of a paper by Dr. Jacques Lamarche, a neuropathologist at the University of Sherbrooke in Québec, Canada. Dr. Lamarche had discovered that the heart of patients with FA contained tiny granules of iron. Dr. Lamarche wanted to explore the role of these iron granules further, so he applied for research funding. The funding agency in Québec, however, did not give the proposal a fundable priority. It was not until 16 years later, after Dr. Massimo Pandolfo and his colleagues identified the FA gene mutation and the FA gene's protein product (called Frataxin after Friedreich's ataxia) was determined to be iron-related, that considerable attention was drawn back to Dr. Lamarche's work.

Dr. Lamarche had begun a systematic assay of iron in hearts of nine patients with FA but found levels within the normal range. Because of the seemingly negative findings, he did not publish these results. In 2006, however, Dr. Koeppen examined the heart iron content of nine additional patients with FA and pooled his new data with those of his friend, Dr. Lamarche. In this FARA-supported investigation, he could confirm that total heart iron remained normal. It was apparent that hearts in FA are not subject to

a global increase of iron. Nevertheless, the iron-carrying protein, ferritin, displayed a shift in its subunits that could only be explained by a small increase in free iron. These findings supported the hypothesis that decreased levels of frataxin lead to abnormal iron handling in the heart. Since frataxin is a mitochondrial protein, researchers began to focus on the presence of abnormal amounts of iron in these tiny power packs of all cells, including those of the heart.

After the work on the heart in FA, Dr. Koeppen began to look at the tissues of the nervous system with similar methods. He examined brain, spinal cord, and dorsal root ganglia. The dentate nucleus of the cerebellum (named after its similarity to developing teeth) was of special interest because it normally contains abundant iron. A FARA-funded study on the dentate nucleus was published in 2007. Dr. Koeppen confirmed that the dentate nucleus was in fact severely affected in FA, and that cellular re-localization of iron was involved. Additional work from his laboratory also suggested that the historical emphasis on the spinal cord in FA is misleading. The critical nervous system lesions are those of the dorsal root ganglia (the main way stations of sensory input to the spinal cord) and the dentate nucleus (the main output station of the cerebellum). In a follow-up study utilizing a new method, X-ray fluorescence, Dr. Koeppen made quantitative measurements of iron, copper, and zinc in the dentate nucleus. In the normal dentate nucleus, these metals occupy separate tissue compartments. In FA, iron, copper, and zinc become admixed. The combination of these metals may be more injurious to the tissues of the dentate nucleus than when each metal acts alone. Dr. Koeppen has now extended his work with X-ray fluorescence to a better understanding of the heart in FA. In his most recent study, funded by FARA's Keith Michael Andrus Award for Cardiac Research, he gained greater insight into the way by which iron becomes toxic to the heart in FA. Specifically, his study revealed that the iron overload occurred in the liquid portion of heart cells, the cytosol, rather than only in mitochondria. This observation is important because it changes the previous concept that disturbed iron metabolism affects only mitochondria. Cytosolic iron excess may offer new opportunities for the therapy of FA heart disease.

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Ride Ataxia Grows Community of Support

By Kyle Bryant

The ride has come a long way in five years, not only in number of participants and dollars raised but in overall support for the effort. In 2012, the Ride program grew from four to six locations due to the efforts of a core group of teammates who plan, organize, communicate with permitting entities, make reservations, recruit volunteers, secure donations, hand out flyers and raise excitement for the ride. Planning for a ride typically starts one year in advance and requires constant communication and work from the local committee. We'd like to highlight the efforts of some of our local leaders:

- *The Ride in Dallas drew the largest number of participants of the entire Ride program, requiring a lot of volunteer recruitment and volunteer hours from our local Dallas families. In addition to volunteer recruitment, the Dallas families baked, canvassed bike shops, secured sponsors and in-kind donations, shopped for supplies, rode, and fundraised. Outback Steakhouse JVP, Mike Yates, led the Dallas effort. He secured a partnership with University of North Texas (the event site) and worked with the city and county on the bike route. The Outback Team promoted the ride like crazy and provided significant fundraising totaling more than \$95,000!*
- *In NorCal, Patrick Kruk and his Outback Steakhouse team have provided relentless support over several years at multiple Northern California fundraising events including the actual ride. Our riders likely consumed more calories than they burned on this ride not only because of the Outback ribs at the end but also because of the plentiful snacks at all the rest stops provided in partnership with UC Davis Dining Services and the Mazeres Family. We count on the strength and enthusiasm of all of our NorCal Families for planning, promotion, and fundraising, which consistently brings in over \$100,000.*



Sam Bridgman gets ready to ride at Ride Ataxia Portland.

- *Our success during our first year in Chicago was due in large part to the leadership of FA mom, Becky Young and Outback JVP, Bob Irons. Becky drove an hour each way three times per week for months to scout the route, meet with municipalities to finalize permits, and secure the event venue. Bob rallied not only his team but also his colleagues at Bonefish Grill and Carrabba's Italian Grill to ride, fundraise, and serve up a post-ride picnic feast.*
- *University of Portland senior, Sam Bridgman, was our breakout leader for the new ride in Portland this year. Sam united several entities including University of Portland, Sauvie Island Academy, Community of Sauvie Island, Multnomah County Sheriff's Office, and Outback Steakhouse. These groups all contributed to a highly successful event in Portland that raised \$55,000! Special thanks to Jim Brown and Jason Bender from Outback Steakhouse for coordinating an awesome post-ride meal.*
- *The strength of our Philadelphia location comes from John Jackson and Charlotte Pizzo who rally their Outback Steak-*

Ride Ataxia 2013 Schedule

RA Dallas (Denton, TX)	March 23
RA NorCal (Davis, CA)	June 1
RA Chicago (Channahon, IL)	July 21
RA Portland (Sauvie Island, OR)	September 21
RA Philly (Blue Bell, PA)	October 13
RA Orlando (Clermont, FL)	November 3



Team Fearless members Grace Haupt and Holly Hedrick celebrate at the Ride Ataxia Philly finish line.

house team as well as Bonefish Grill and Carrabba's Italian Grill to provide an amazing spread that erased any hardship of cycling in the rain this year. With this ride, John and Charlotte have built a platform for our Northeast Families to have a great time and raise funds consistently reaching \$140,000-plus.

• Jennifer Shaw and Julie Strasser began cycling in 2010 as participants in Ride Ataxia Tampa. When the ride moved closer to home in Orlando in 2011, they jumped to the forefront of planning to coordinate ride promotion, ridership, volunteerism, and sponsor support. We also enjoyed a new and growing partnership with the local Outback Steakhouse and Carrabba's Italian Grill headed up by Tony Alonge, Jennifer Alsip, and Virgil Morar.

Over the past five years, Ride Ataxia has raised \$2 million for FA research (See A Review of FARA's Research Grant Program for more information about the latest grant funded by Ride Ataxia proceeds.) As Ride Ataxia has grown in number of participants and dollars raised, the greatest impact has come from local families and friends making it their priority to make this program succeed. These dedicated folks and hundreds of others have helped us make a tangible impact on research and will continue to do so as we journey ever closer to a treatment and a cure. ●



Members of the FA community gather for a Family photo following Ride Ataxia Chicago.

Continued from page 7

Dr. Koeppen's observations are examples of the progress in FA research over the past five years. His discoveries were made possible by families of patients who succumbed to FA. The close relatives agreed to donate tissue samples from their deceased family member for research. Beyond his own research, Dr. Koeppen has also provided FA tissues to other scientists from various disciplines who study FA. Among them are Drs. Mark Payne at the University of Indiana, Miriam Cnop at the University of Brussels, Grazia Isaya at the Mayo Clinic, Edward Grabczyk at Louisiana State University, Paul Hahn at the Kirby Center of Molecular Ophthalmology in Philadelphia, Marek Napierala at MD Anderson Cancer Center in Houston TX, and Erika Becker at the University of Sydney, Australia.

Many investigators now explore FA by the methods of molecular biology, tissue culture, imaging, and pathology of heart, brain, eyes, dorsal root ganglia, spinal cord, and nerves. These varied methods have one common goal, namely, to determine how deficiency of a small protein called frataxin affects so many organ systems. What is the common denominator? Dr. Koeppen contends that FA researchers and physicians caring for FA patients must join hands to conquer the disease. He asserts that clinical management must be multidisciplinary: Neurologists must understand heart disease and diabetes mellitus; cardiologists must gain knowledge about the grave neurological manifestations of FA; orthopedists operating on FA patients with advancing scoliosis must be aware of FA heart disease and the risk that it presents during and after the operation. Anesthesiologists should also realize the added risks due to FA cardiomyopathy and diabetes mellitus. Dr. Koeppen believes that basic science must remain an essential part of FA research while we test promising new drugs in clinical trials. He readily proclaims: "it must be all of us."

For further information on organ and tissue donation and the benefit to research contact Dr. Koeppen by e-mail at arnulf.koeppen@med.va.gov; by phone at 518-626-6377; or fax at 518-626-6369. His address is VA Medical Center, Research Service (151), 113 Holland Ave, Albany, N.Y. 12208. ●

The FARA Advocate is brought to you by:

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A Parent's Perspective on FA Research

By John Jacquin

It has been 14 years since Laura was diagnosed with Friedreich's Ataxia. In that time my daughter has gone from performing gymnastics, dancing, riding her bike, climbing trees, swimming, skiing, drawing - and walking - to life in a wheelchair with poor vision, speech problems, and no ability to write. Yet there is hope ... over the past 10 years there has been tremendous progress made in understanding how to cure FA. I want to share a couple brief stories with you.

"I can see colors again"

These are Laura's words. Many of you know that during her last year at Berry College, Laura lost most of her field of vision and became color blind. Last winter she got her color vision back! Why? Because last October she began participation in a clinical trial with an experimental drug. In fact, Laura's ability to discern or delineate objects has improved, she has better trunk strength, her speech (and volume) is better, and the neuropathy - shown by the purplish coloration in her lower legs - has lessened - and her legs are no longer so cold. Due to the generous donations over the years, this new pharmaceutical was developed in California and tested coast to coast in trials - and it has REVERSED some aspects of Laura's progressive neuromuscular disease.

The mice lived - all of them

Mice genetically modified to have Friedreich's ataxia usually die of cardiac failure in a few months. However, after receiving an experimental gene therapy, the FA mice whose health had been declining for seven weeks lived. These mice did not only live, they thrived. All the mice with FA recovered to the health level of the wild mice in the control group without Friedreich's ataxia. Again, your donations helped to support the research that developed a gene replacement therapy, where the synthetically manufactured gene replaces the frataxin in the mice. We need more financial support to move this forward into human clinical trials.

Let's go to Italy

Repligen Corp., a small biopharmaceutical company in Massachu-



John with his daughter, Laura, and wife, Patti, at the FARA Energy Ball.

setts, has licensed a new class of drug called **histone deacetylase (HDAC) inhibitors** from Scripps Research Institute in California. Animal studies showed that this new therapy could specifically treat the underlying molecular cause of Friedreich's ataxia. But the FDA needs a lot of information, and patients with a progressive disease like FA are fighting the clock. So enter Turino, Italy, where a phase 1 clinical trial is underway with 20 adults that have FA receiving a single dose (at subsequently higher levels) of the HDAC drug. The trial is evaluating drug safety and how the drug interacts with the body. Favorable clinical results could pave the way for later-stage trials both in Europe and the United States for the treatment of FA. Meanwhile, Repligen recently announced that it has a follow-up version of HDAC that may be even more effective. HDAC inhibitors were developed through the support of research funded by donations to FARA and the MDA. Let's keep the support going!

With Laura as the inspiration and enthusiastic team captain, the Jacquin Family has raised over \$140,000 for FA research in ten years through the participation of Team Laura in numerous fundraising events such as FARA's Ride Ataxia Philadelphia and the Muscular Dystrophy Association's local walks. •



Outback Steakhouse President, Jeff Smith with his wife, Sheree, and FARA Co-Founders, Ron and Raychel Bartek.

FARA Energy Ball For a Cure Raises Record Funds

By Felicia DeRosa

The 4th annual FARA Energy Ball welcomed over 700 guests and raised a record-breaking \$1.5 million (gross) in support of FA research. Due to the planning committee's promotion of the event throughout Tampa (such as radio PSAs, billboards, regular e-blasts, and print media) as well as efforts to educate and engage friends and business partners, the event has continued to grow its proceeds for research in the face of hard economic times. The evening was creatively lit with a "stormy night giving way to sunny day" theme inspired by the bright light of promising FA research leading us through difficult times, and a nod to Honorary Chair Tod Lieweke, who is best known for his role as CEO of the Tampa Bay Lightning Professional Hockey Team.

The bright light of promising FA research was the focus of the Scientific Symposium hosted by FARA's partners at the University of South Florida (USF) in their new Center for Learning and Medical Simulation two days prior to the Ball. Thanks to USF's expertise in live stream technology, FARA was able to share late-breaking research updates presented by Drs. Mark Payne, Guy Miller and Hélène Puccio beyond the standing room-only 200+ live crowd to 500 online viewers and more than 900 additional viewers through Ustream. Dr. Payne, of University of Indiana, presented on cardiomyopathy in FA and his research on TAT frataxin as a treatment approach to cardiac disease in FA. Dr. Guy Miller, of Edison Pharmaceuticals, discussed emerging therapies

to treat mitochondrial diseases and along with Dr. Zesiewicz announced their intention to do a study in FA with EPI-743. And Dr. Puccio, of the Institut de Génétique et de Biologie Moléculaire et Cellulaire, presented her exciting discoveries with gene therapy in the rescue of cardiac function in the FA mouse model. It was a powerful evening of research progress announcements, and this energy carried over to the gala event.

The Energy Ball highlights included amazing food by A La Carte Event Pavilion, the National Anthem sung by American Idol finalist Shannon Magrane, high energy live entertainment by The Soul Power Review, and numerous exclusive experiences in the live and silent auction secured by the hardworking and dynamic committee. This year, the stand-out moment of the evening for many was the Fund a Cure. Each year, guests are given the opportunity to make a contribution to FA research, specifically the Collaborative Clinical Research Network in FA. The CCRN in FA is where people with FA participate in critical FA research through the natural history study, giving blood samples or cheek swabs, or enrolling in clinical trials.

Following a moving video produced by Broad Reach Television featuring some of our New Jersey FA families, Emcee Wendy Ryan and auctioneer Scott Robertson kicked off the 2012 Fund a Cure. This year pledges were tracked in real time and the top six contributing tables were displayed on large screens in the ballroom, adding some friendly competition to pledging. Also, as an added feature, Honorary Chair Tod Lieweke offered an official Tampa Bay Lightning Jersey and an invitation to a progressive wine party at the Tampa Bay Times Forum culminating in the Tampa Bay Lightning's locker room for anyone contributing \$1,000 or more to the Fund a Cure. All of these elements converged to create a perfect storm of bidding and generosity in support of the CCRN in FA. The energy in the room was palpable as the contributions quickly passed last year's fund total and then surged to double it. The bidding continued for several minutes and culminated in over \$180,000 raised for clinical research in FA! Thanks to Tod & Tara Lieweke and all of the donors who contributed to more than triple last year's Fund a Cure total.

A special note of gratitude to our planning committee and sponsors whose commitment to FARA continue to yield the resources needed to accelerate research. ●

SAVE THE DATE! FARA Energy Ball September 5-7, 2013



Long time FARA supporters, Doug and Kathy Rothschild and Tom and Dede Bradley.

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FARA Names Paul & Mary Jacobs Partners of the Year

By Felicia DeRosa

Too often we underestimate the power of a touch, a smile, a kind word, a listening ear, an honest compliment, or the smallest act of caring, all of which have the potential to turn a life around.

— Leo Buscaglia

At FARA, we believe in the power of our partners to impact the course and speed of FA research and thereby turn many lives around. We are a small staff focused on a rare disease, but with the backing of our partners, we become a large presence and a strong force. This year's Partner of the Year Award recipients demonstrate the power of one couple to make a significant difference in the lives of many. For Paul and Mary Jacobs, what may have started as an act of caring for their friends and former neighbors, the Avery Family, has grown into a self-driven movement to give FARA the resources we need to affect change in the research landscape.

The Jacobs have been top level Presenting Sponsors at the Energy Ball for several years, contributing significant financial resources in support of FA research. They have also sponsored, attended, and volunteered at other Tampa-based FARA events such as Pull for the Cure Clay Shoot and Ace for the Cure Tennis Tournament. The couple act as Development Advisors for FARA, are founding members of the FARA Energy Ball planning committee, and participate in all local Ride Ataxia events. FARA Executive Director, Jen Farmer, says, "Whenever I see Paul or Mary helping with the set-up at an event, I breathe a sigh of relief because they are a constant in our fundraising efforts and events." FARA President, Ron Bartek, echoes, "Whenever, I see Paul and Mary before an event, I, too, breathe a sigh of relief, because I know they'll be bringing an army of friends and supporters to that event."

The Jacobs have made it their mission to educate their friends and colleagues about FARA's work and make them active contributors to the effort to treat and cure FA. They hosted a gathering in their home with FARA Ambassador, Kyle Bryant, just so their friends



Paul and Mary Jacobs

could get an understanding of FA, FARA, and why it was so important to support the cause. The Jacobs' friends indeed took up the charge. Some friends, like members of the Raymond M. Craig Family Foundation, attend fundraising events, contribute as sponsors, and donate high caliber live auction items to the FARA Energy Ball. Other friends lend their time and talent to better the organization. For example, Paul and Mary's cycling enthusiast friends, Matt and Melissa Lazinski, have traveled from Tampa to several Ride Ataxias. They recruit riders and help with event logistics such as marking the course prior to the ride. The couple's friend, Jennifer Vickery, runs her own PR firm and volunteered her services in 2012 to garner media attention at local Ride events, significantly impacting awareness of Friedreich's ataxia. And Paul and Mary's friend, Chef Walt Wickman, hosted two seafood festival fundraisers at his restaurant, Olde Bay Café, in Dunedin this year for FARA. The Jacobs family, of course, rolled up their sleeves and worked tirelessly at the festivals on FARA's behalf.

We often say, "It takes a community to find a cure." Paul and Mary Jacobs give of themselves to "turn lives around," and rally their community to do so as well. FARA is honored to call Paul and Mary friends and partners and enthusiastically celebrate them as our Partners of the Year. ●

2012 Friend and Ally Awards Celebrate Selfless Spirit of Giving

By Felicia DeRosa

Working in FARA fundraising often gives one the opportunity to see the best of humanity — families empowered to make a difference in the research landscape on behalf of their loved one and communities that rally around that family. Occasionally, we meet someone who has no prior connection to FA and is still moved to give. Kyle and I had an opportunity to meet such a person when we approached a homeowner along the Ride Ataxia Dallas route to ask permission to use his property for a rest stop. This man never heard of Friedreich's ataxia before, but listened to us, welcomed us in, and asked for nothing in return. With this year's Friend & Ally Awards, we celebrate four people who embody that selfless spirit of giving to FARA and the FA community.

Rod Clingaman — Working Behind the Scenes for So Many He's Never Met. Rod Clingaman is an employee of EDS, an HP company. Six years ago, he was asked by a colleague to assist with a database project for FARA to develop and maintain FARA's critically important patient registry. Anytime there is a technological issue with the database, Rod immediately looks into the issue, sets about correcting it and always signs off on a follow-up email wishing the recipient a "Fantastic Day." Recently, the patient registry underwent a significant upgrade to capture additional information about registrants to help inform research studies. Rod was the man behind all of these upgrades, and he spent countless programming hours on the project. Rod works tirelessly on behalf of FARA behind the scenes acting as a Friend and Ally to help so many with FA that he's never met.

Tom & Amy Leobold — Giving a Voice to a Rare Disease. If you've seen a FARA video recently, you know the amazing filming, editing, and producing done by Tom & Amy Leobold. The Leobolds are the dynamic duo that make up Broad Reach Television and are responsible for FARA's Introductory Video, all four

of the FARA Energy Ball videos, and pieces featuring sports celebrities like Rondé Barber, James Blake, and John Isner. Every year Tom and Amy travel north from their home in Florida to spend a weekend interviewing and filming FA families and FARA representatives. They have constructed several beautiful pieces that educate and communicate the challenges of living with FA as well as the magnetic and determined spirit of our FA families. The Leobolds have shared their talent and resources to give a voice to the FA rare disease cause, making them true Friends, Allies, and teammates of FARA and all people living with FA.

Ava Forney — Energy Ball Event Coordinator Extraordinary. Rarely do you come across a person who can simultaneously manage the high volume of event details and be a creative visionary behind the look, feel, and flow of that same gala event. FARA is really fortunate to have met such a person in Ava Forney, the Event Coordinator for the FARA Energy Ball the past three years. Ava helps dream up the creative design that adds to the high-energy feel of the gala, and she also helps secure sponsorship, contributes auction items, and keeps calm even while working late into the early morning during a demanding pre-event week. Ava's ability as a masterful event planner has been a significant factor in the continued success of the Energy Ball. The Ball is responsible for a third of FARA's annual budget and consequently helps accelerate FARA's research funding. Sharing her creative vision and detail management to make the FARA Energy Ball such a success, Ava helped provide critical resources in support of FA research and has been a continued Friend and Ally to FARA. •

FARA Store

For yourself, your family and friends, or to have customized FARA items at fundraising events, visit the FARA store!
www.cureFAstore.com.
FARA caps, polo and t-shirts, wristbands, etc.



Organizers Share Highlights from Successful Grassroots Parties

By Marilyn Downing

A diagnosis of FA is a difficult and lonely place, but several of our families discovered strength and a platform for fundraising and awareness through fundraising events. Each of these featured fundraisers has adapted the idea of a family party in their own unique way and found great fundraising success in support of FARA's mission.



Swing Away at FA had another great turnout from the FA community enjoying great food and games.

One proud and determined mom

Hannah is in her element, at the microphone, thanking everyone for coming. Her mother is beaming, but wondering why once again at their third annual event, nine-year-old Hannah went to the face painting booth first! Hannah's party is called Swing Away at FA. Candy Stacks, the proud mother, wasn't planning to do the event again. When her youngest child was also diagnosed with FA in May, life seemed overwhelming. But her friends jumped in, and encouraged and supported the family—and on October 13, raised an amazing \$32,000. There were bounce houses, a raffle, silent auction, face painting, T-shirt sales, a barbecue that included smoked pork, and the infamous wiffle ball game. At the end of the day, Candy said, "After the success this year, no doubt we'll do it again".

If only all of us had an Uncle Neal

With a passion for skiing, Neal & Libby Lacativo have an annual party in Truckee, a ski area in California just 20 miles from the Nevada border. Here you will find a community of about 70 people who travel to Truckee to ski, but who also look forward to Lacativo's fundraiser for Friedreich's ataxia. The local businesses donate lift tickets, restaurant gift certificates, admission tickets to public entertainment and more for the popular raffle and silent auction. Kyle Bryant is Neal's nephew and is the guest of honor when he proudly travels to Truckee to attend each year. Outback Restaurant has generously provided the dinners and the Lacativos cover the additional expenses, so all the money raised is donated to FARA. The amount, which totaled about \$15,000 in 2012, has increased each year.

Six years and growing

Things have changed for the Welsh family in the past six years, but the biggest change is in Brendan, who has grown from a reluctant participant to a young man with the confidence to stand before the crowd, talk about FARA's mission, and thank the audience for being a part of this mission. The Bash includes tents, appetizers kindly provided by Outback Steakhouse, a cookout, 50/50 and basket raffles, and music by a popular local band. More than \$20,000 was raised by friends and family at the Bash in the Backyard VI this year. Brendan's mother, Francine, said that they feel increasingly connected to the FA community, or the FAMily. Francine says, "It's not just about our family anymore."



Eilish, Francine, Brendan, and Pete Welsh welcoming the community to their Bash in the Backyard.

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Making music, hiking, galloping, and golfing

It's important to know your audience when you plan a fundraiser. Dylan McDonnell knew his people loved music — and his father, David, was part of a classic rock band. The McDonnell Music Festival was born in Queensbury, NY in 2008, raising more than \$50,000 for FARA since then. Dylan says, "It gets easier, bigger and better all around every year." His support comes from a loving community that holds additional fundraisers for FARA, including the Goblin Gallop, two golf tournaments, and an Adirondack hike, all in honor of Dylan. ●

The FARA Ambassador Group

Speaking at an upcoming fundraiser or event?

Don't know what to say? Want to try it out before you get in front of the crowd? The FARA Ambassador Program seeks to prepare people in the FA community to be ready to represent themselves and FARA when the opportunity presents itself. The Ambassadors' group meets on a regular basis over Webex to discuss any upcoming speaking opportunities and allow that person to practice what they want to say before they get in front of the crowd. The FARA Ambassador Program is also a great forum for sharing ideas on living with FA, FARA updates, and developing strong friendships within the community. If you would like to be a part of the FARA Ambassador Program and attend regular meetings over Webex, please email Erin O'Neil (lana3158@aol.com) for information on how to join.

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On Your Mark... Get Set... Go...

By Jamie Young

"...You never may know the success you may have until you try," says Katie Hopkins, Race for Matt and Grace event organizer. This was particularly true this fall during an exceptionally active FARA grassroots fundraising season. A number of athletic events raised a great amount of research funds and awareness but also demonstrated a show of strength by FA families who overcame a challenging diagnosis and took fundraising action.

Finding Ways to Help

Two years ago in an email, Julie Myers, mother of Justin Myers, wrote, "We are new to the FARA world. With what it feels like literally nothing else we can do, we want to find ways to help encourage awareness and raise money to support FA research and help find a cure for this disease." Julie's fundraising path started by writing letters to her community in support of her husband Jeff's participation with Team FARA in the St. Louis Rock n' Roll Half Marathon. She received great support and decided that she was ready to take the next step and plan a race of her own. More than 300 participated in Slim's Journey: FARA 5K, an event that brought together community volunteers, FAmilies, sponsors, and donors to raise over \$22,000. After reaching out to FAPG and the FA community, Julie received great support from seven additional FAmilies: The Mahers, Jones, Sciortinos, Devines, Henrys, Wisemans, and Kruses.



Group shot of the biggest turnout yet at Race for Matt & Grace.



Some of the 300 participants receiving their finishing medals at Slims Journey 5K.

Joining Forces

Katie Hopkins, an avid 5K runner, was participating in monthly races and decided "I CAN DO THIS." From there Katie, with help from her brother, organized the first Race for Grace in honor of their cousin Grace Hopkins at Colt State Park in Rhode Island in 2010. Katie learned that there are people who are always willing to help, and the key ingredient to a successful event is a strong committee. That strong committee now also includes The DiIorios: Jack, Sally Ann, and Matt. The DiIorios have been huge supporters, providing planning, volunteers, participants and fundraising support for the event. Now called The Race for Matt and Grace, the event is complete with a 5K race, raffle, and an after party with great food and music to celebrate their efforts which have raised more than \$50,000 in three years.

Sharing the Journey

Norm, Debra, Donovan, and Grandma Marilyn Simpson along with family friend Sally Braid are not new to the FARA fundraising calendar. They have created an athletic fundraiser that has had growing success over the past nine years. What started with one friend participating in a local triathlon on behalf of Donovan grew to a TEAM of triathlon athletes, volunteers and generous donors. For the Simpsons, the hardest part about starting the fundraising process was going public with the news and allowing the community to witness the changes along with their family. However, Norm and Debra also realized that events like Team

Continued from page 17

Donovan gave their community a platform to offer support through writing letters, fundraising, and event participation. The Simpsons have not only let their community in on their journey with FA but continually engage them by writing personal thank you letters and giving updates on Donovan and FA research. Norm states, "There is a raw pain in each of us as parents and learning how to harness it can be a powerful tool! After all we are fighting for our children!!" ●



Members of Team Donovan 2010 gather for an annual Team photo prior to the Triathlon.

Memorials

From January 2012 to October 2012, FARA received over \$20,000 in memory of the following individuals:

Adam Main, Albert Golanec, Benjamin Orth, Bil Brothers, Carol DeFilippo, Carolyn Irvin, Dallas Gendall, David Schaus, Dawn Kent, Diana Taylor, Faye Thomas, Frances Baxter, Gladys Lacativo, James Dour, Jennifer Alexander, John Andresen, Keith Andrus, Kenneth Wayne, Kevin Fleming, Kevin Maher, Leonard Graham, Margaret Hopkins, Marilyn Shrauger, Merrill Rushin, Phillip Bennett, Phillip C. Collins, Ray Endreny, Robert Bogucki, Sal DeRosa, Shelly Reed, and Thomas Barnett.

Thank you to all who chose to remember a loved one with a donation to FARA.

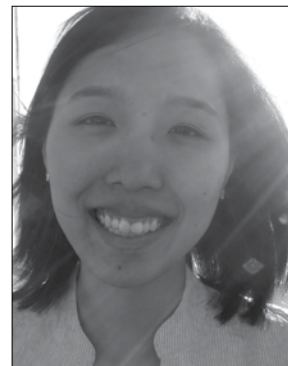
To request envelopes to be used for memorials, please contact FARA at info@cureFA.org.

FARA hires new staff

By Evelyn Wu

If you've called FARA headquarters recently, you may have heard someone new answering the phone—me!

In October 2012, I joined FARA as the new Administrative and Development Assistant. In this role, I will manage FARA's gift processing and support the work of the FARA team. I came to FARA with four years of non-profit health care experience at Planned Parenthood Southeastern PA and a background in Biological Sciences from Wellesley College. While I don't have a personal connection to FA, my interest in nonprofit management and public health led me to join the FARA family. I feel so lucky to be part of this overwhelmingly supportive and positive community! I look forward to working with you as together we get closer to a cure and treatments for FA. ●



Evelyn Wu

Grassroots Grid

2012 Events	Event Organizer	Location	In Honor of
NORTHEAST			
FARA Benefit Dance	Beth Drake	Franklin, VA	Jason Drake
Valentine's Day Dance	Becky Chase	Fairhaven, MA	Jade Perry
Tasting Party	The Passport To Excellence	Edison, NJ	John Ryan
Spark Hope Cocktail Party	Jean Walsh	Beverly, MA	Walsh Family
We Sing for Team Donovan	We Sing For The World	White Plains, NY	Donovan Simpson
NJ Seaside Stride	Karen O'Brien	Seaside Heights, NJ	NJ FA Families
Rockin for a Cure	Erin O'Neil & Richie & Maria Courier	Wakefield, MA	FARA & The FA Project
Outback Charity Golf Classic	Cornell & Diedre Barnett	Waldorf, MD	FARA & All FA families
Fundraiser for FARA	John Cernosek	Bowie, MD	John Cernosek
Chase-Perry Yard Sale	Becky Chase	Fairhaven, MA	Jade Perry
Cogan's Heroes Century Ride	Patrick Cogan	Essex, MA	Patrick Cogan
Outback Steakhouse Luncheon	Golanec, Simpson, and Richard Families	White Plains, NY	Kaela Golanec, Donovan Simpson, & Albert Richard
Outback Steakhouse Luncheon	Outback Steakhouse Springfield	Springfield, VA	FARA & All FA families
Welsh Family Bash in the Backyard	Francine Welsh	Harrisburg, PA	Brendan Welsh
Kickin for Kids	Alicia Strong	Plainville, CT	Brian Bianchi
Fuzzy Buzzy Golf Tournament	Paul & Maureen Stanieich	Windham, NH	Erin O'Neil
TEAM FARA — Team Donovan- Jarden Westchester Triathlon	Simpson Family & Sally Braid	Rye, NY	Donovan Simpson
McDonnell Music Festival	David McDonnell	Queensbury, NY	Dylan McDonnell
Race for Matt and Grace	Katie Hopkins & The Dilorio Family	Colt State Park, RI	Matt Dilorio & Grace Hopkins
Heineken with a Heart	Glen Bianchi & Mary Caruso	Newington, CT	Brian Bianchi and Sam & Alex Bode
CrossFit KOP Anniversary Party Fundraiser	Aimee Lyons	Bridgeport, PA	Kyle Bryant
TEAM FARA — Gold Coast Triathlon & Mossman Sprint	Megaera Regan	Long Island, NY & Philadelphia, PA	Gracie Hopkins
TEAM FARA — Century Bike Ride	Richie Currier	Essex, MA	FARA & The FA Project
TEAM FARA — Rock 'n Roll Half Marathon	Felicia DeRosa	Philadelphia, PA	FARA & All FA families
TEAM FARA — ING NYC Marathon	Steve Wisinski, Lori Pitta, Brian Schutter, John Lagedrost, Margaret Hay, Patrick Hammill	New York, NY	Jack DeWitt, Donovan Simpson, Joanna Lagedrost, Don Royer
SOUTHEAST			
Friedreich's Ataxia BINGO & Pampered Chef BINGO	Dawn Lambert	Palm Harbor, FL	Gavin Lambert
Olde Bay Cafe Anniversary Fundraiser	Olde Bay Cafe	Dunedin, FL	FARA & All FA families
Anytime Fitness Golf Scramble	Dawn Lambert	Palm Harbor, FL	Gavin Lambert
Bake Sale	FSC Occupational Therapy Assistant Students	Jacksonville, FL	Team Alex & Ariel
Kneel United Prayer Concert	Vicki Kennedy	Tampa, FL	FARA & All FA families
Swing Away at FA	Candy Stacks	Dawsonville, GA	Hannah & Austin Stacks
Chris's Fight Car & Bike Show	Amanda Laird	Cape City, KY	FARA & All FA families
Pull for a Cure Clay Shoot	Mike Mezrah & TBSC	Land O Lakes, FL	FARA & All FA families
Ace for a Cure	Sandy Callaghan & Roger Cypriano	Tampa, FL	FARA & All FA families
TEAM FARA — Walt Disney World Marathon	Susan Piroth	Orlando, FL	Dylan & Keith O'Brien
WEST			
Truckee Fundraiser	Neal & Libby Lacativo	Truckee, CA	Kyle Bryant
Race 4 Results & Rabbit Run	Carrie Laird	Bakersfield, CA	Jerod Laird
St. Catherine's Annual Jog-A-Thon	Greg Jones	Anaheim, CA	Chelsea Lane
Izzy's Angels Concert	Zoe Penston	Alameda, CA	Izzy Pentson
Ride Ataxia MTZ Fundraiser	Angela Lacativo Greene	Martinez, CA	Kyle Bryant
Friends of Jerod Reverse Draw Dinner	Carrie Laird	Bakersfield, CA	Jerod Laird
Century 21 Golf Tournament	Brian & Carolyn Lamascus	Rancho Cucamonga, CA	Joshua Lamascus
Stephanie's Hope Holiday Boutique	The Magness Family	Santa Clarita, CA	Stephanie Magness
Outback Steakhouse Luncheon	Susie Esqueda	Tacoma, WA	Roman DeCrote
Outback Steakhouse Luncheon	Paul & Susan Konanz	Rohnert Park, CA	Brianne Konanz
Horizon High School's Classroom Donation Contest	Maya Rodich	Scottsdale, AZ	FARA & All FA families
Acker Family Dinner/ Dance	Beejay Acker	Merin County, CA	Kiela Acker
TEAM FARA — Tour De Palm Springs	FARA	Palm Springs, CA	FARA & All FA families
TEAM FARA — Reno-Tahoe Odyssey (RTO)	Sean Baumstark	Reno, NV	FARA & All FA families
TEAM FARA — Team Adirondack- High Sierra Trail Hike	Mike DelSignore	Sequoia National Park, CA	Dylan McDonnell
MIDWEST			
Lone Star Benefit Bash	Jerry Russell, Vince Palasota & Performance Award Center	Flower Mound, TX	Robbi and Becca Van Shoick
Folf for FARA	Ashley Day	Libby, MT	Kyle Bryant
Outback Steakhouse Golf	Andy Shallit	San Antonio, TX	FARA & All FA families
FA Woodstock	Paula & Tom Hook	LaPorte, IN	Carli Hanson & Kati Hook
Wildfire Kids Triathlon	Mike Newman & Allison Moore	Missoula, MT	Dylan & Sienna Helms
Hole Out for a Cure	Rick Peters	Peoria, IL	Rick & Jeff Peters
Slim's Journey 5k Run/ Walk	Julie Myers	Warrenton, MO	Justin Myers
Hayden's Hope Warrior Run	Sarah Despenas	Mason City, IA	Hayden Despenas
Outback Steakhouse Luncheon	Kimberly Trentham	Columbia, MO	Karsen Trentham
Trentham Family Christmas Concert	Kimberly Trentham	Centralia, MO	Karsen Trentham
FA Thanksgiving Dinner/Dance	Tammy Luebbe & John Spencer	Cincinnati, OH	Evan Luebbe and The Spencer Family
TEAM FARA — Ironman Wisconsin	Holly Tripp	Madison, WI	FARA & All FA families
TEAM FARA — Chicago Marathon	Vicki Peer & Doug Finck	Chicago, IL	Jenn, Jamie, Carli Hanson, Kati Hook
CANADA			
A Night of Nostalgia Fashion Show	Penelope Alefantis & Litsa Condon	Calgary, Alberta	Penelope Alefantis

Fundraising



Jade and her partner-in-crime fundraise for FARA at their Annual Yard Sale in Fairhaven, MA.



Izzy Penston and a friend welcome guests to Izzy's Angels Concert in Alameda, CA.



Izzy and Zoe Penston thank their community for supporting the event.



Donovan, Kaela, and Tom get ready to raise funds for FARA at the Outback Steakhouse Luncheon in White Plains, NY.



Albert, Emily and Donovan anticipate delicious food by Outback Steakhouse.



A banner welcomes FA families from around the world to FA Woodstock in LaPorte, IN.

Fundraising



The Hook Family's Flying H Ranch provides sun and fun at FA Woodstock!



Holly Tripp in her Team FARA gear as she gets ready for the Ironman Wisconsin.



Erin O'Neil and Paul Stanieich share a hug after another successful Fuzzy Buzzy golf tournament in Windham, NH.



Team Crizzy, the top fundraising team for Ride Ataxia 2012, gets excited to ride in Orlando on behalf of Isabel and Christian Maugee.



Members of Team Donovan celebrate at the Westchester Triathlon finish line in Rye, NY.



Just all smiles and sunshine for the Swing Away at FA teams!



Viki Peer gets ready to run at her Team FARA event at the Chicago Marathon.



Kati Hook and Carli Hanson with Doug Finck as he completes another Chicago Marathon on behalf of Team FARA.



Energy Ball Honorary Chairs, Tod and Tara Lieweke with American Idol finalist Shannon Magrane.

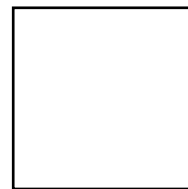


Team Tara for FARA came out in record numbers to ride and volunteer for Ride Ataxia Orlando.

What is Friedreich's Ataxia?

Friedreich's Ataxia is a debilitating, life shortening, degenerative neuro-muscular disorder. Onset of symptoms can vary from childhood to adulthood and can include loss of coordination (ataxia) in the arms and legs; energy deprivation and muscle loss; vision impairment, hearing loss, and slurred speech; aggressive scoliosis (curvature of the spine); diabetes mellitus; and a serious heart condition. While the mental capabilities of people with FA remain completely intact, the progressive loss of coordination and muscle strength in FA leads to motor incapacitation and the full-time use of a wheelchair. There is currently no treatment or cure for FA. FARA is a 501 (c) (3) tax exempt, non-profit organization dedicated to supporting research that will improve the quality and length of life for those diagnosed with Friedreich's Ataxia and will lead to treatments that eliminate its symptoms.

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WINTER 2012-2013

the Advocate

Your connection to the Friedreich's Ataxia Research Alliance



www.cureFA.org

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